

Project Data Sphere & MCC patient Registry: From Promise to Prototype to Patient

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Disclosures

- David Miller
 - I have received honoraria from Pfizer, Merck Sharpe & Dome and Regeneron for contributions to advisory boards
- Michael K Wong
 - I have received honoraria from EMD-Serono, Pfizer, Merck, and Regeneron for contributions to advisory boards.
 - I receive research support from Merck, Kartos and Bristol Myers Squibb.

Unmet Need

- Clinical outcomes research limited to large administrative data sets or single-institution databases
- Uncertainty in presentation, outcomes and patterns of failure
- Uncertainty in sequencing of available treatments
- Limited approvals for advanced MCC
 - Future approvals for advanced MCC limited by “n”

Origins



Origins

EXPERT CONSENSUS STATEMENT

The biology and treatment of Merkel cell carcinoma: current understanding and research priorities

*Paul W. Harms¹, Kelly L. Harms², Patrick S. Moore³, James A. DeCaprio^{1,4},
Paul Nghiem⁵, Michael K. K. Wong⁶ and Isaac Brownell^{1,7*}, on behalf of the
International Workshop on Merkel Cell Carcinoma Research (IWMCC) Working Group*

“The clinical management of MCC would be improved by the creation of a **collaborative infrastructure that enables formal information sharing** and the rapid design and implementation of clinical trials”

Origins

The screenshot shows the homepage of the Project Data Sphere website. At the top, there is a blue navigation bar with white text containing links for HOME, ABOUT, ACCESS DATA, SHARE DATA, RESOURCES, and NEWS. To the right of the navigation bar is a search icon and a "Sign In" link. Below the navigation bar is the Project Data Sphere logo, which features a blue sphere composed of small white plus signs. To the right of the logo is a green button labeled "APPLY FOR ACCESS". The main content area contains a large text block describing the organization: "A non-profit enterprise devoted to cancer clinical trial data-transparency, data-sharing and data-analysis founded by the *CEO Roundtable on Cancer's Life Sciences Consortium*". Below this text is a graphic element consisting of a light blue arrow pointing to the right, containing the text "Learn more about this collaboration" and a "VIEW THE DATA" button. At the bottom of the graphic, it says "Made possible with support from the Robert Wood Johnson Foundation". There are also three small circular icons at the bottom of the graphic.

HOME ABOUT ACCESS DATA SHARE DATA RESOURCES NEWS

SEARCH Sign In

Project Data Sphere®

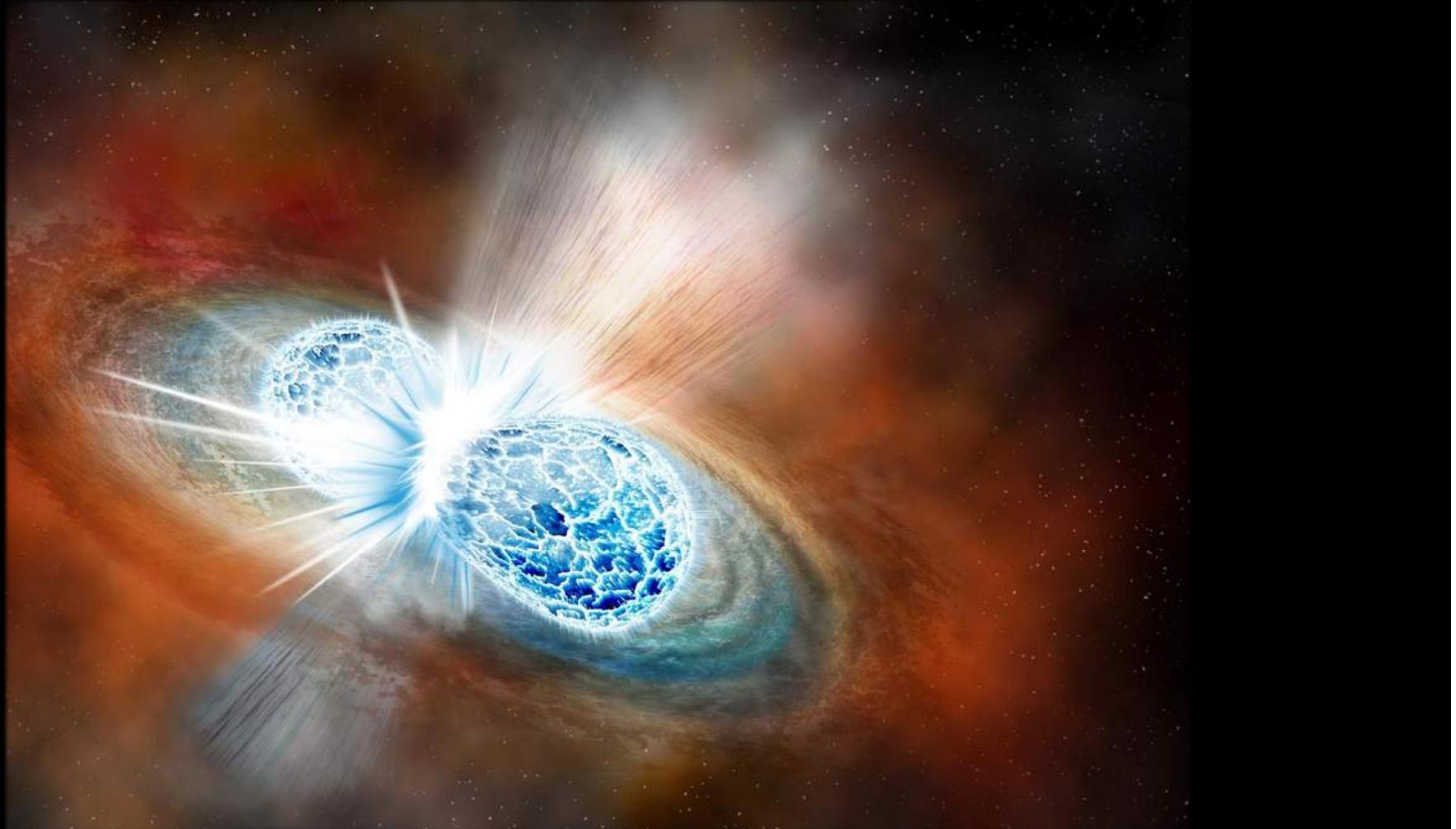
APPLY FOR ACCESS

A non-profit enterprise devoted to cancer clinical trial data-transparency, data-sharing and data-analysis founded by the *CEO Roundtable on Cancer's Life Sciences Consortium*

Learn more about this collaboration

VIEW THE DATA

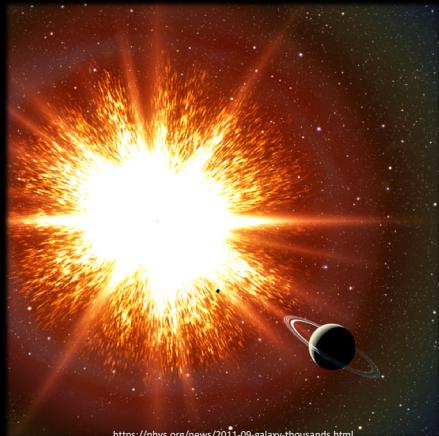
Made possible with support from the Robert Wood Johnson Foundation



<https://www.independent.co.uk/life-style/gadgets-and-tech/news/stellar-explosion-gravitational-waves-einstein-gold-platinum-jewellery-universe-expanding-a8004846.html>

Task Force - Members

- Academia
- NIH/NCI
- FDA
- Sponsor
- Project Data Sphere

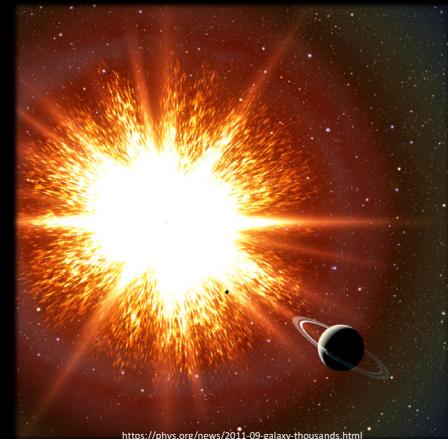


<https://phys.org/news/2011-09-galaxy-thousands.html>

Task Force - Members

- **Academia**

- Michael Wong (MDACC)
- David Miller (MGH)
- Paul Nghiem (UW)
- Ken Tsai (Moffit)
- Chris Bichakjian (Mich)
- Kelly Harms (Mich)
- Kristina LaChance (UW)
- Lauren Haydu (MDACC)
- Vishal Patel (GW)



<https://phys.org/news/2011-09-galaxy-thousands.html>

Task Force – Current & Near-Term Objectives

- Identify the objectives and applications of an MCC Patient Registry
- Select and Develop a Data Collection Instrument
- Develop and Execute an Implementation Strategy

Task Force - Objectives



Merkel Cell Carcinoma Registry
David M. Miller MD PhD, Michael K. Wong MD PhD
October 8, 2018

Executive Summary

The Merkel Cell Carcinoma (MCC) Patient Registry is a national multi-institutional collaborative effort that will prospectively follow and record outcomes and events in MCC patients. MCC is the prototypical rare tumor, and this Registry will trail blaze new methodologies that will enable multiple investigators to examine real world outcome data in real time. Deliverables from the Registry include: (i) precise patient stratification into risk categories, (ii) identification of best practices, (iii) revelations about optimal sequence and combinations therapies, (iv) uncovering low incidence toxicities and (v) the generation of novel testable hypotheses. Importantly, the Registry offers a way forward in the yet-unsolved dilemma of drug development for rare tumors since the Registry's design will allow for the creation of highly defined patient-level data that can be used as a robust comparator for single arm Phase I-II clinical trials.

The MCC Task Force comprises members from academic medical centers, the drug industry, the NIH and FDA. Project Data Sphere, LLC provides a secure, open-access data sharing platform and comprehensive support to optimize research performance and ensure rigorous and timely results. The Registry is currently in the planning stage but will be based on a REDCap database integrated into the host institution's electronic medical record. We plan to have the first patient accessioned on Project Data Sphere's data platform in Q1 of 2019.

Objectives And Applications

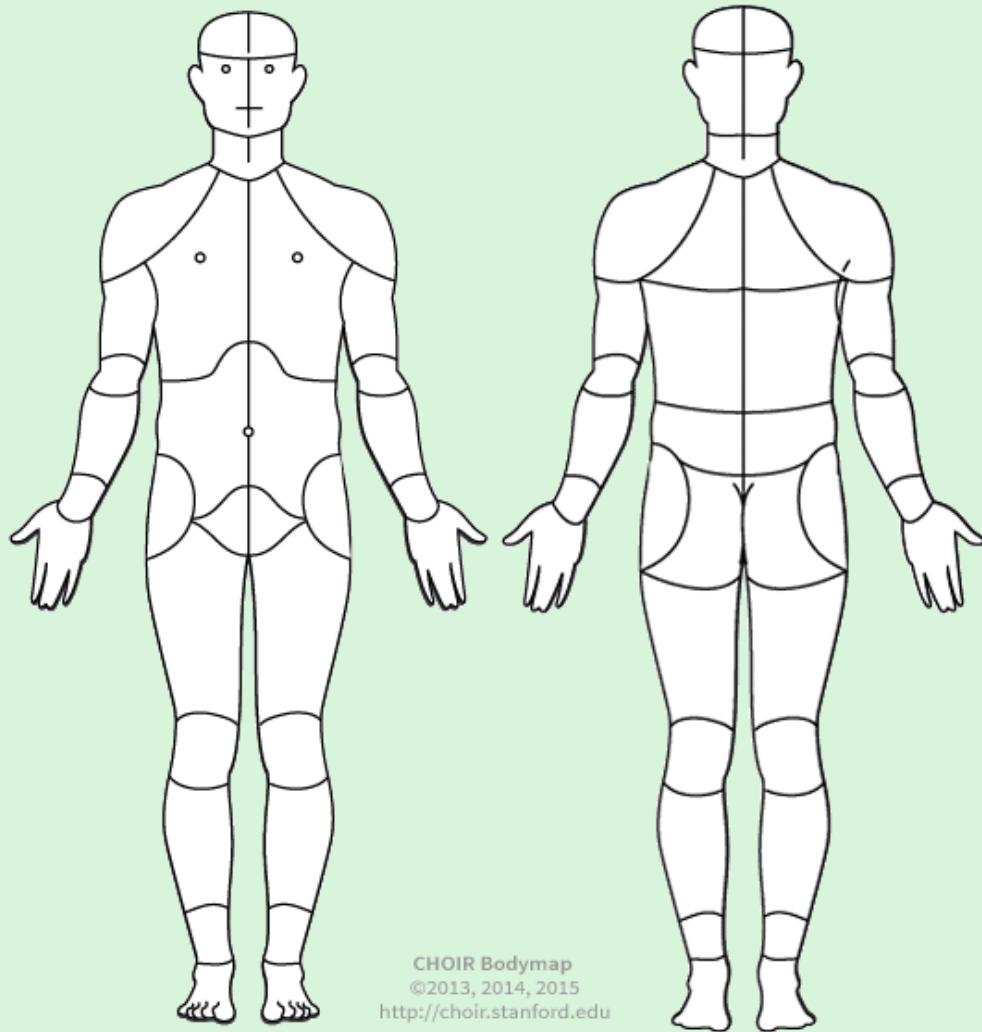
- **Natural History Study**
 - Precisely characterize the presentation and natural history of Merkel Cell Carcinoma
 - Identify best practices in MCC
 - Identify accurate covariates that are prognostic in MCC
- **Patient Level Data As Real World Evidence for Drug Development**
 - Function as external data set
 - Tool for safety assessment
 - RWE to support sBLA/sNDA

Data Capture Tool – REDCap



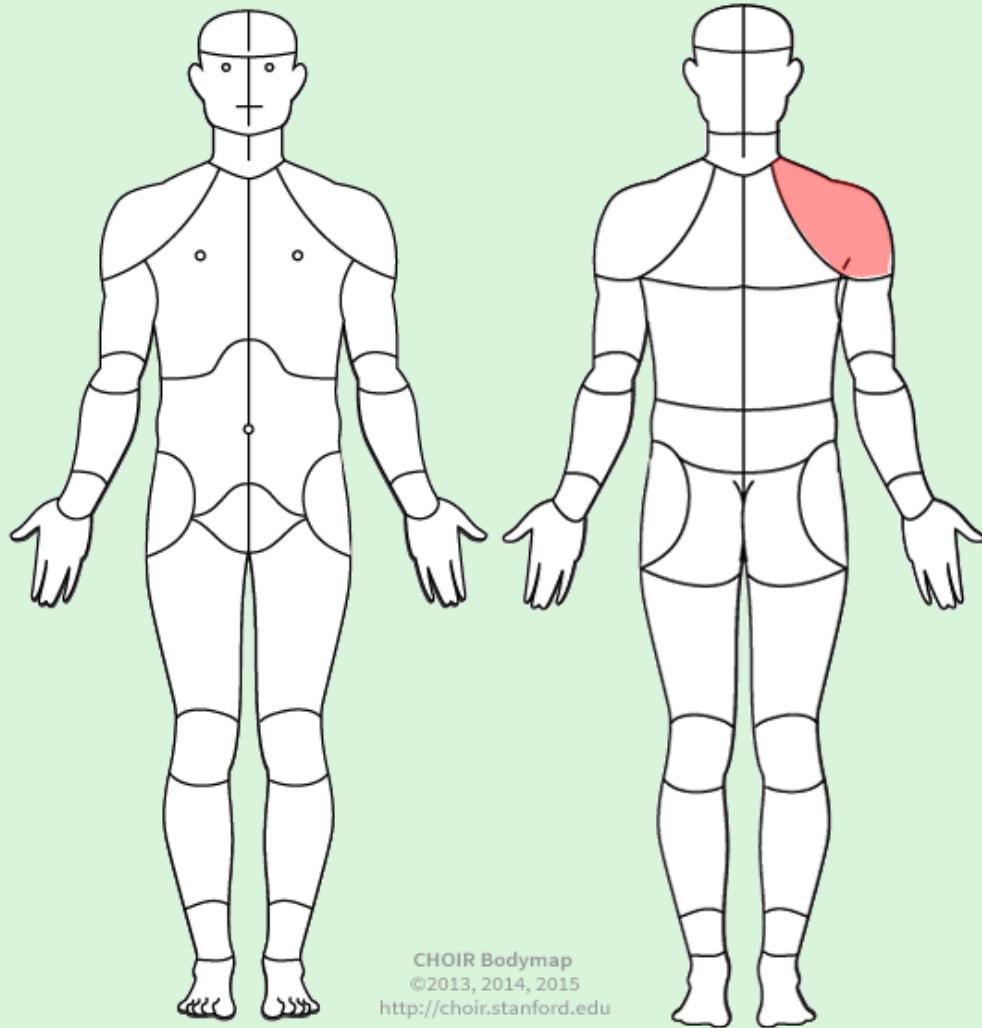
- REDCap is a secure web application for building and managing databases
- It can be used to collect virtually any type of data, including
 - HIPAA-compliant environments
 - 21 CFR Part 11
- The REDCap consortium is a vast support network of collaborators

The image on the LEFT side of your screen is the FRONT of the body. The image on the RIGHT side of your screen is the BACK of the body.



CHOIR Bodymap
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<http://choir.stanford.edu>

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Logged in as dm883 | Log out

- [My Projects](#)
- [Project Home](#)
- [Project Setup](#)

Project status: Production

Data Collection

- [Record Status Dashboard](#)
- [Add / Edit Records](#)
- [**Study ID \(record_id\) 1_4**](#)

Data Collection Instruments:

- Protected Health Information
- Non PHI Clinical Characteristics Of The Patient (PMH, Social Hx, etc)
- Clinical Characteristics of the Initial Presentation - Features of the primary lesion, location, In-transits, etc.** + []
- Imaging - Initial Staging (3) + []
- Surgical Therapy - Initial Excision + []
- Lymph Node Evaluation - Initial Work Up + []
- Metastases - Initial Staging
- Pathology - Initial Staging + []
- Staging - Initial
- Radiation Therapy (2) + []
- Medical Anti-Neoplastic Therapy
- Surgical Therapy - Additional (i.e. after initial staging)

Registry of Merkel Cell Carcinoma Patients

Actions: [Download PDF of instrument\(s\)](#) [Share instrument in the Library](#) [VIDEO: Basic data](#) [Save & Exit Form](#) [Save & Stay](#) [Cancel](#)

Clinical Characteristics of the Initial Presentation - Features of the primary lesion, location, in-transits, etc.

Current instance: 1 Data Access Group: [No Assignment] [?](#)

Editing existing Study ID (record_id) 1_4

| | |
|--|--|
| Study ID (record_id) | 1_4 |
| Number of Primary Tumors | |
| How many primary tumors did the patient have at presentation? | 0 |
| * must provide value | Potential for communication with EHRs, e.g. EPIC |
| Upload Photos reset | |
| Description of the Primary Lesion | |
| Does the patient have a primary skin lesion/primary cutaneous MCC? | <input type="radio"/> No (0) <input checked="" type="radio"/> Yes (1) |
| (prim_skin_yn) | Of note, roughly 10-15% of patients present with MCC of unknown primary |
| reset | |
| Is a photo of the primary cutaneous lesion available to review and upload? | <input type="radio"/> No (0) <input checked="" type="radio"/> Yes (1) |
| (image_prim_mcc) | * must provide value |
| reset | |
| Image of Lesion (image) | 1_4 MCC left forehead 2.pdf (1.25 MB) |

Implementation Strategy

- Task 1: Build Consensus Data Fields
- Task 2: Rollout and Scalability Plan

Task 1: Consensus Data Fields

| Data Collection Instruments | |
|--|--------|
| Instrument name | Fields |
| Protected Health Information | 10 |
| Non PHI Clinical Characteristics Of The Patient (PMH, Social Hx, etc) | 60 |
| Clinical Characteristics of the Initial Presentation - Features of the primary lesion, I | 22 |
| Imaging - Initial Staging | 11 |
| Surgical Therapy - Initial Excision | 10 |
| Lymph Node Evaluation - Initial Work Up | 26 |
| Metastases - Initial Staging | 11 |
| Pathology - Initial Staging | 67 |
| Staging - Initial | 55 |
| Radiation Therapy | 18 |
| Medical Anti-Neoplastic Therapy | 29 |
| Surgical Therapy - Additional (i.e. after initial staging) | 8 |
| Adverse Events | 18 |
| Subject Status | 13 |
| Clinical Benefit/Response | 12 |

| Data Collection Instruments | |
|---|--------|
| Instrument name | Fields |
| Recurrence | 19 |
| Progressive Disease | 17 |
| Lab Results - MCC Polyoma Virus Serum Oncoprotein Antibody Test | 5 |
| Lab Results - Hematological | 14 |
| Lab Results - Electrolytes/Renal | 12 |
| Lab Results - Cardiac | 6 |
| Lab Results - Endocrine | 7 |
| Lab Results - LDH | 4 |
| Imaging - Restaging/Ongoing Follow up | 12 |
| Pathology - Followup | 36 |
| Palliative Care/Hospice | 3 |
| Death Details | 17 |
| Clinical And Translational Research - Protocol Documentation | 3 |
| Translational Research - Biospecimen Collection Documentation | 8 |
| Calculated Fields For Preliminary Statistics | 15 |

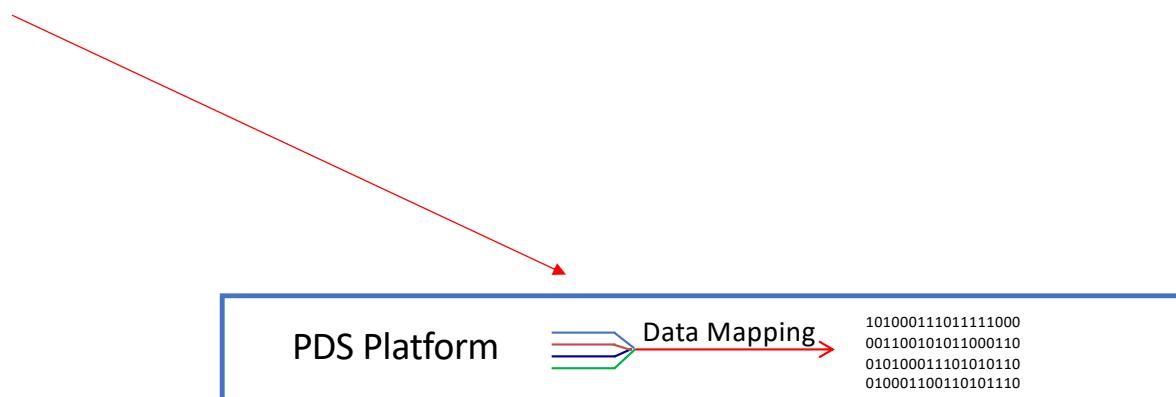
Task 2: Rollout and Scalability Plan

- Phase I – Prospective Capture

Task 2: Rollout and Scalability Plan

Tiered Model for Data Access

- I. Closed Access
- II. Limited Access
- III. Open Access



Next Steps

- Test Beta Version
- Develop Data Quality Plan
- Develop Data Usage Agreements



Support

- MCC Community
- Project Data Sphere
 - Research Administration
 - Data Management
 - PDS vast network

Acknowledgements

- Task Force Members
- PDS
- FDA
- Merck KGaA, Darmstadt, Germany
- MMIG